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Public summary of opinion on orphan designation

Allogeneic cultured postnatal thymus-derived tissue for the treatment of severe combined immunodeficiency due to FOXN1 deficiency

On 26 February 2019, orphan designation (EU/3/19/2137) was granted by the European Commission to Enzyvant Therapeutics Ireland Limited, Ireland, for allogeneic cultured postnatal thymus-derived tissue (also known as RVT-802) for the treatment of severe combined immunodeficiency due to FOXN1 deficiency.

What is severe combined immunodeficiency due to FOXN1 deficiency?

Severe combined immunodeficiency due to FOXN1 deficiency is an inherited disorder where the patient is unable to fight infections due to the lack of a type of white blood cells called T cells. The disorder is caused by mutations (changes) in the gene for FOXN1. FOXN1 is involved in the formation of the thymus gland (a gland below the breastbone that helps T cells to develop properly).

Severe combined immunodeficiency due to FOXN1 deficiency is a long-term debilitating and life-threatening condition due to recurrent and severe infections.

What is the estimated number of patients affected by the condition?

At the time of designation, severe combined immunodeficiency due to FOXN1 deficiency affected less than 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 500 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of application for orphan designation, there was no satisfactory treatment for severe combined immunodeficiency due to FOXN1 deficiency authorised in the EU. Patients were managed with supportive care. Some patients had received experimental blood stem cell or bone marrow transplants.

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 518,400,000 (Eurostat 2019).

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How is this medicine expected to work?

The medicine comprises slices of tissue from a donor's thymus gland. The donor tissue is processed in a laboratory so that it is compatible with the patient's body and is then inserted into the patient's body by surgery. This is expected to help patients produce T cells and fight infections.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with severe combined immunodeficiency due to FOXN1 deficiency were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for severe combined immunodeficiency due to FOXN1 deficiency or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 24 January 2019 recommending the granting of this designation.

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on the EMA website.

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Allogeneic cultured postnatal thymus-derived tissue	Treatment of severe combined immunodeficiency due to FOXN1 deficiency
Bulgarian	Алогеннна култивирана постнатална тимусна тъкан	Лечение на тежък комбиниран имунодефицит, причинен от дефицит на протеина FOXN1
Croatian	Alogeni proizvod dobiven iz kulture postnatalnog tkiva timusa	Liječenje teške kombinirane imunodeficijencije zbog nedostatka FOXN1
Czech	Alogenní kultivovaná postnatální tkáň thymu	Léčba těžké kombinované imunodeficience způsobené deficiencí FOXN1
Danish	Allogent dyrket postnatalt præparat deriveret fra thymusvæv	Behandling af svær kombineret immunodeficiens forårsaget af FOXN1 deficiens
Dutch	Weefsel verkregen uit allogeen, gekweekt, postnatale thymus	Behandeling van ernstige, gecombineerde immuundeficiëntie gebaseerd op FOXN1 deficiëntie
Estonian	Allogeenne kultiveeritud postnataalse tüümuse kude	FOXN1 puudulikkusest tingitud raskekujulise kombineeritud immuunpuudulikkuse ravi
Finnish	Allogeenisesta, viljellystä syntymänjälkeisestä kateenkorvakudoksesta peräisin oleva valmiste	Vaikean kombinoidun, FOXN1:n puutteesta johtuvan, immuunivajauksen hoito
French	Produit dérivé de culture de tissu de thymus postnatal allogénique	Traitement du syndrome du déficit immunitaire combiné sévère du à une déficience en FOXN1
German	Allogenes Kulturprodukt kultiviert aus postnatalem Thymusgewebe	Behandlung des schweren kombinierten Immundefizits verursacht durch Defizit von FOXN1
Greek	Αλλογενής καλλιεργημένος ιστός από μεταγεννητικό ιστό θύμου	Θεραπεία της βαριάς συνδυασμένης ανοσοανεπάρκειας λόγω έλλειψης FOXN1
Hungarian	Allogén, tenyésztett, postnatalis csecsemőmirigyből származó szövet	FOXN1 elégtelenség következtében kialakuló, súlyos, kombinált immunhiányos állapot kezelése
Italian	Prodotto derivato dal tessuto timico postnatale coltivato allogenico	Trattamento dell'immunodeficienza severa combinata da deficit di FOXN1
Latvian	Alogēni, kultivēti, postnatāli no aizkrūtes dziedzera iegūti audi	FOXN1 nepietiekamības izraisīta smaga, kombinēta imūndeficīta ārstēšana
Lithuanian	Alogeninis postnataliai iš užkrūčio liaukos išskirtas dirbtinai išaugintas audinys	Sunkaus bendro imunodeficito, kurį lemia FOXN1 stoka, gydymas

[1] At the time of designation

Maltese	Tessut derivat mit-timus alloġeniku kkultivat, postnatali	Il-kura tal-immunodefiċjenza kkombinata u severa minħabba d-defiċjenza ta' FOXN1
Polish	Allogeniczna kultywowana tkanka poporodowej grasicy	Leczenie ciężkiego złożonego niedoboru odporności powodowanego niedoborem FOXN1
Portuguese	Derivado da cultura de tecido tímico pós-natal alogénico	Tratamento da imunodeficiência combinada grave devido à deficiência de FOXN1
Romanian	Produs derivat din cultură de țesut timic postnatal alogenic	Tratamentul imunodeficienţei severe combinate datorate deficitului de FOXN1
Slovak	Alogénny liek kultivovaný v tkanivách postnatálneho týmusu	Liečba závažnej kombinovanej imunodeficiencie následkom FOXN1 deficiencie
Slovenian	Alogena kultura iz tkiva poporodnega priželjca	Zdravljenje hude kombinirane imunske deficience zaradi pomanjkanja FOXN1
Spanish	Producto alogénico derivado tisular de timo postnatal cultivado	Tratmiento de la inmunodeficiencia combinada grave variante FOXN1
Swedish	Odlad allogen postnatal tymusvävnad	Behandling av svår kombinerad immunbrist på grund av FOXN1-brist
Norwegian	Allogent vev fra thymus dyrket postnatalt	Behandling av alvorlig kombinert immunsvikt som følge av FOXN1 mangel
Icelandic	Ósamgena ræktaður vefur afleiddur úr hóstarkirtli eftir fæðingu	Meðferð á alvarlegum samsettum ónæmisbresti af völdum skorts á FOXN1